Briefing for the Public Petitions Committee

Petition Number: PE1401

Main Petitioner: Lesley Loeliger on behalf of PNH Scotland and Professor Peter Hillmen on behalf of the PNH Alliance.

Subject: Access to therapy for paroxysmal nocturnal haemoglobinuria.

Calling on the Scottish Parliament to urge the Scottish Government to review the mechanism and methodology used by the Scottish Medicines Consortium to appraise medicines for rare diseases and to instruct the Chief Medical Officer to revise the criteria by which health boards assess Individual Patient Treatment Requests in order to improve access to therapy for patients with paroxysmal nocturnal haemoglobinuria.

Introduction: paroxysmal nocturnal haemoglobinuria (PNH)

PNH is a rare acquired genetic disease affecting approximately 1 in 500,000 people. Due to a deficiency in a particular protein (glycosyl phosphatidylinositol (GPI)-linked complement inhibitory protein C59) production of red blood cells is impaired and they are more easily destroyed than normal. Symptoms can include anaemia, fatigue, weakness, erectile dysfunction, kidney damage, shortness of breath and an increased heart rate. PNH may also result in the formation of potentially fatal blood clots. Median survival time from diagnosis is estimated to be 15 years.

Treatments for PNH

PNH patients may require blood transfusions to help manage the disease and a long term cure may be achieved with bone marrow transplantation, although this is not possible in most patients due to lack of availability of a suitable donor. Another treatment recently made available is eculizumab (Soliris®), a drug which blocks the body’s defence system from prematurely destroying red blood cells. It is delivered via intravenous infusion and must be administered once every two weeks. Eculizumab (Soliris®) was appraised by the Scottish Medicines Consortium (SMC) in 2010 (reviewed in 2011) with the resulting conclusion being that it “is not recommended for use within NHS Scotland”.

Access to treatments on the NHS

This petition, PE1401, is particularly concerned with the processes for assessing medicines for use in NHS Scotland and for submitting and assessing individual patient treatment requests. These processes are discussed in the petition briefing for petition PE1398.
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